

## Highly Specialised Technologies Evaluation Committee Meeting

**Minutes:** Confirmed

**Date and Time:** Wednesday 20 January 2016, 10am to 5pm

**Venue:** National Institute for Health and Care Excellence  
Level 1A, City Tower  
Piccadilly Plaza  
Manchester  
M1 4BT

<b>Present:</b>	1. Peter Jackson, Chair 2. Sotiris Antoniou 3. Ron Akehurst 4. Trevor Cole 5. Sarah Davis 6. Jonathan Howell 7. Jeremy Manuel 8. Francis Pang 9. Linn Phipps 10. Mark Sheehan 11. Anthony Wierzbicki	Present for all notes Present for all notes Present for all notes Present for notes 12 to 29 Present for all notes Present for notes 12 to 29
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### In attendance:

Meindert Boysen	Programme Director, National Institute for Health and Care Excellence	Present for all notes
Sheela Upadhyaya	Associate Director, National Institute for Health and Care Excellence	Present for all notes
Jenna Dilkes	Project Manager, National Institute for Health and Care Excellence	Present for all notes
Leanne Wakefield	Project Manager, National Institute for Health and Care Excellence	Present for all notes
Linda Landells	Technical Adviser, National Institute for Health and Care Excellence	Present for all notes
Mary Hughes	Technical Analyst,	Present for notes 1 to 17

	National Institute for Health and Clinical Excellence	
Martyn Burke	Technical Analyst, National Institute for Health and Clinical Excellence	Present for notes 18 to 29
Maiwenn Al	Kleijnen Systematic Reviews	Present for notes 18 to 27
Anoukh van Giessan	Kleijnen Systematic Reviews	Present for notes 1 to 15
Manuela Joore	Kleijnen Systematic Reviews	Present for notes 1 to 15
Rob Riemsma	Kleijnen Systematic Reviews	Present for notes 1 to 15 and x to x
Emma Harvey	Alexion	Present for notes 18 to 27
Scott Johnson	Alexion	Present for notes 1 to 15 and 18 to 27
Radhika Tripuraneni	Alexion	Present for notes 1 to 15
Nick Bishop	Clinical Expert	Present for notes 18 to 27
Patrick Deegan	Clinical Expert	Present for notes 1 to 15
Simon Jones	Clinical Expert	Present for notes 1 to 15
Zulf Mughal	Clinical Expert	Present for notes 18 to 27
Elaine Murphy	Clinical Expert	Present for notes 1 to 15
Nick Shaw	Clinical Expert	Present for notes 18 to 27
Amjad Akhtar	Patient Expert	Present for notes 1 to 15
Stuart Lancaster	Patient Expert	Present for notes 1 to 15
Sophie Thomas	Patient Expert	Present for notes 1 to 15
Lindsay Weaver	Patient Expert	Present for notes 18 to 27
Edmund Jessop	NHS England	Present for notes 1 to 15 and 18 to 27

**Non-public observers:**

Helen Barnett	Editor, NICE	Present for all notes
Chloe Kastoryano	Public Involvement Adviser, NICE	Present for all notes

## Notes

### Welcome

1. The Chair welcomed all members of the Committee and other attendees present to the meeting. The Chair reviewed the agenda and timescales for the meeting, which included the Evaluations of **Sebelipase alfa for treating lysosomal acid lipase deficiency** and **Asfotase alfa for treating paediatric-onset hypophosphatasia**
2. The Chair informed the Committee of the non-public observers at this meeting: Helen Barnett and Chloe Kastoryano.
3. Apologies were received from Vincent Kirkbride, Lesley Stewart and Steve Brennan

### Any other Business

4. None

### Notes from the last meeting

5. The minutes from the meeting which took place on 17 November 2015 were agreed.

### Evaluation of sebelipase alfa for treating lysosomal acid lipase deficiency

#### Part 1 – Open session

6. The Chair welcomed the invited experts: Patrick Deegan, Simon Jones, Elaine Murphy, Amjad Akhtar, Stuart Lancaster, Sophie Thomas, Edmund Jessop, Anoukh van Giessan, Manuela Joore and Rob Riemsma to the meeting and they introduced themselves to the Committee.
7. The Chair welcomed representatives from Alexion Pharma UK to the meeting.
8. The Chair asked all Committee members to declare any relevant interests
  - 8.1. Peter Jackson, Sotiris Antoniou, Ron Akehurst, Trevor Cole, Sarah Davis, Jonathan Howell, Jeremy Manuel and Mark Sheehan all declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of sebelipase alfa for treating lysosomal acid lipase deficiency
  - 8.2. Linn Phipps declared a personal non-pecuniary interest due to her role as Patient & Public Voice Representative on NHS England's CPAG (Clinical Priorities Advisory Group)
    - 8.2.1. It was agreed that this declaration would not prevent Linn Phipps from participating in the meeting.
  - 8.2. Anthony Wierzbicki declared a non-personal pecuniary interest for his role as site clinical trial investigator for lipid-lowering drug therapies -

Amgen (evolucumab); Merck Sharpe and Dohme (Anecetrapib); Site lead for clinical registry - Chesi (Lipoprotein lipase deficiency)

8.3.1 It was agreed that this declaration would prevent Anthony Wierzbicki from participating in this section of the meeting.

8.4 Francis Pang declared a non-specific personal pecuniary interest due to his employment with a healthcare company (which has no relationship to the technology under evaluation)

8.4.1 It was agreed that this declaration would prevent Francis Pang from participating in this section of the meeting.

9. The Chair asked all NICE Staff to declare any relevant interests.

9.1. All declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of sebelipase alfa for treating lysosomal acid lipase deficiency

10. The Chair asked all other invited guests assessment group/ERG and invited experts, not including observers) to declare their relevant interests.

10.1. Amjad Akhtar, Stuart Lancaster, Sophie Thomas, Edmund Jessop, Anoukh van Giessan, Manuela Joore and Rob Riemsma declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of sebelipase alfa for treating lysosomal acid lipase deficiency

10.2 Patrick Deegan, Simon Jones and Elaine Murphy declared personal pecuniary interests due to their roles as Clinical Trial Investigators for sebelipase alfa for treating lysosomal acid lipase deficiency

10.3 It was agreed that this declaration would not prevent Patrick Deegan, Simon Jones and Elaine Murphy from participating in the meeting.

11. The Chair introduced the lead team, Sotiris Antoniou, Linn Phipps and Sarah Davis who gave presentations on the clinical effectiveness, patient perspective and cost effectiveness of sebelipase alfa for treating lysosomal acid lipase deficiency.

12. The Committee then discussed the clinical effectiveness, patient perspective and cost effectiveness of sebelipase alfa for treating lysosomal acid lipase deficiency on the basis of the evidence before them, and potential equality issues raised in this evaluation. They sought clarification and advice from the experts present

13. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
14. The Chair explained that "representatives of the press and other members of the public be excluded from the remainder of this meeting having regard to the confidential nature of the business to be transacted, publicity on which would be prejudicial to the public interest" (Section 1(2) Public Bodies (Admission to Meetings) Act 1960)" and all public attendees left the meeting.
15. The Chair then thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

## **Part 2 – Closed session**

16. Discussion on confidential information continued.
17. The Committee instructed the technical team to prepare the Evaluation Consultation Document (ECD) in line with their decisions.

## **Evaluation of asfotase alfa for treating paediatric-onset hypophosphatasia**

### **Part 1 – Open session**

18. The Chair welcomed the invited experts: Maiwenn Al, Nick Bishop, Edmund Jessop, Zulf Mughal, Rob Riemsma, Nick Shaw and Lindsay Weaver to the meeting and they introduced themselves to the Committee.
19. The Chair welcomed representatives from Alexion to the meeting.
20. The Chair asked all Committee members to declare any relevant interests
  - 20.1. Peter Jackson, Ron Akehurst, Sotiris Antoniou, Steve Brennan, Sarah Davis, Jonathan Howell, Jeremy Manuel, Mark Sheehan and all declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of asfotase alfa for treating paediatric-onset hypophosphatasia.
  - 20.2. Trevor Cole declared a personal non-financial interest as a clinician for patient with hypophosphatasia.
    - 20.2.1. It was agreed that this declaration would not prevent Trevor Cole from participating in this section of the meeting.
  - 20.3. Francis Pang declared a non-specific personal pecuniary interest due to his employment by a healthcare company which has no relationship to the technology under evaluation
    - 20.3.1. It was agreed that this declaration would prevent Francis Pang from participating in this section of the meeting.

20.4. Linn Phipps declared a personal non-pecuniary interest due to her role as Patient & Public Voice Representative on NHS England's CPAG (Clinical Priorities Advisory Group).

20.4.1. It was agreed that this declaration would not prevent Linn Phipps from participating in this section of the meeting

20.5. Anthony Wierzbicki declared a non-personal pecuniary interest as the site clinical trial investigator for lipid-lowering drug therapies for Amgen and Merck Sharp & Dohme. Also site lead for clinical registry for Chiesi.

20.5.1. It was agreed that this declaration would not prevent Anthony Wierzbicki from participating in this section of the meeting.

21. The Chair asked all NICE Staff to declare any relevant interests.

21.1. All declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of asfotase alfa for treating paediatric-onset hypophosphatasia.

22. The Chair asked all other invited guests assessment group/ERG and invited experts, (not including observers) to declare their relevant interests.

22.1. All declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of asfotase alfa for treating paediatric-onset hypophosphatasia.

23. The Chair introduced the key themes arising from the consultation responses to the Evaluation Consultation Document (ECD) received from consultees, commentators and through the NICE website.

24. The Committee then discussed the clinical effectiveness, patient perspective and cost effectiveness of asfotase alfa for treating paediatric-onset hypophosphatasia on the basis of the evidence before them, and potential equality issues raised in this evaluation. They sought clarification and advice from the experts present:

25. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.

26. The Chair explained that "representatives of the press and other members of the public be excluded from the remainder of this meeting having regard to the confidential nature of the business to be transacted, publicity on which would be prejudicial to the public interest" (Section 1(2) Public Bodies (Admission to Meetings) Act 1960" and all public attendees left the meeting.

27. The Chair then thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

## **Part 2 – Closed session**

28. Discussion on confidential information continued. This information was supplied by Alexion.
29. The Committee instructed the technical team to prepare the Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED) in line with their decisions.

## **Date, time and venue of the next meeting**

30. Wednesday 17 February 2016 10am till 2pm at Friends' Meeting House, 6 Mount Street, Manchester, M2 5NS